Estimating the Societal Economic Burden of Gene and Cell Therapy-Relevant Diseases in Germany: A Model-Based Burden-of-Illness Framework

R. Mumm[4], J. Thiesen[4], A. Backes[4], B. Slavinskaite-Saare[3], V. Zu Rhein[3], J. Gansau[1], C. Gallus[1], D. Häckl[4], C. Elsner[2,3], J. Weber[1]

[1]Berlin Institute of Health at Charité (BIH), Strategic Science Management, Berlin, Germany; [2]University of Lübeck, Lübeck, Germany; [3]PricewaterhouseCoopers WPG GmbH, Frankfurt/Main, Germany; [4]WIG2 Institute for Health Economics and Health Services Research, Leipzig, Germany

EE31

Background & objective

Gene and cell therapies (GCTs) aim to modify disease durably or curatively. Their value is hard to capture in German Health Technology Assessment: no added benefit was established for 54% of orphan-drug research questions assessed under AMNOG in 2011–2023[1]. A systematic, cross-disease estimate of the societal burden of GCT-relevant diseases in Germany does not yet exist.

Objective: To develop and apply a model-based burden-of-illness (BOI) framework to estimate the societal economic burden of diseases for which GCTs are, or could become, relevant.

Annual societal cost of GCT-relevant diseases in Germany

€136.0 bn

base case; range €116.8–184.6 bn

€1,194.8 bn over 10 years (discounted, 3% p.a.)

Methods: four-step, model-based BOI framework (societal, prevalence-based perspective)

Approach: For most GCT-relevant indications no German burden-of-illness study exists. We therefore calculated the full societal cost for **one well-documented use case per disease group**, transferred it to the other indications of that group via their **health-state utility value weights**, and scaled it to Germany with **prevalence data**.

1 Identify indications

21 GCT-relevant indications from literature, experts and AI screening, in 3 groups: **rare diseases**, **oncology**, **high prevalence** (+4 top-mortality cancers).

2 Select use cases

One use case per group, chosen by **GCT relevance** and **data availability**: haemophilia A (rare), NSCLC (oncology), type 1 diabetes (high prevalence).

3 Calculate BOI

Direct and indirect costs from national and international studies, transferred to Germany; price year 2025. Intangible burden as HRQoL/DALYs only (not monetised).

4 Extrapolate & project

Utility-weighted cost imputation to all indications of a group, combined with German prevalence data; discounted 5- and 10-year projection (3% p.a.).

Cost imputation: $C_i = C_{UC} \times (u_{UC} / u_i)$ **Group cost:** $C_y = \Sigma (n_i \times C_i)$ **Scenarios:** pessimistic (u_{min}) · base case (mean) · optimistic (u_{max})

C = annual cost per patient; u = health-state utility value (lower utility → higher imputed cost); n = prevalence; UC = use case; i = indication. Rare diseases: two cost profiles (haemophilia A/B; Duchenne muscular dystrophy for LCA, SMA, retinitis pigmentosa, sickle cell disease).

Use cases: societal BOI of one representative indication per group

Rare diseases

Haemophilia A[2-4]

€1.51 bn per year

€356,000 per patient (avg.)

- 4,240 patients (500 mild · 1,158 moderate · 2,582 severe)
- Per patient: €88,400 - €413,700 (depending on severity); severe = 71% of total costs
- HRQoL 0.78 · 0.69 · 0.67 (mild → severe)

Oncological diseases

NSCLC[5-8]

€1.88 bn per year

€22,582 per patient (avg.)

- 83,054 patients (5-year prevalence, 2023)
- Per patient: €19,317 direct (86%) · €3,265 indirect (14%)
- HRQoL 0.72 disease-free · 0.62 recurrence · 0.67 metastatic

Diseases with high prevalence

Type 1 diabetes[9-12]

€2.35 bn per year

€6,320 per patient (avg.)

- 372,412 patients (31,781 under 18)
- Per patient: €4,496.00 direct (71%) · €1,820 indirect (29%)
- ≈41,500 DALYs, peak at age 50-54

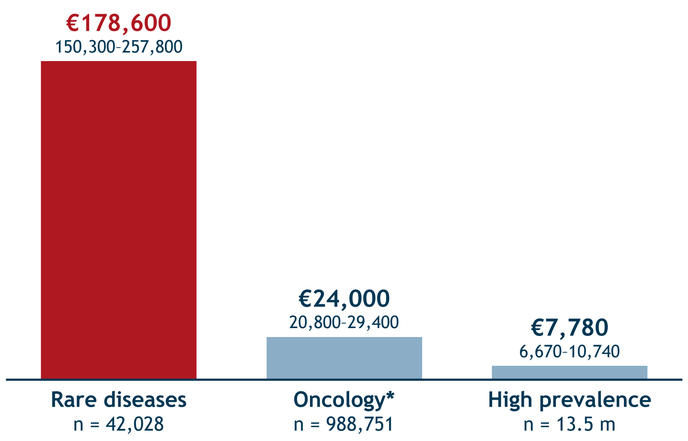
Use case extrapolation by group

1. **Use-case BOI:** annual cost per patient C_{UC} as model input
2. **Cost imputation** to each indication of the group: $C_i = C_{UC} \times u_{UC} / u_i$
3. **Average cost per patient:** $C_{ø} = \Sigma(n_i \times C_i) / \Sigma n_i$
4. **Annual total societal cost:** $C_y = \Sigma(n_i \times C_i)$
5. **Long-term cost** (5–10 years): $C_{Long} = C_{ø} \times \Sigma\, 1/(1+r)^t$

MAIN RESULTS

1 Average cost per patient

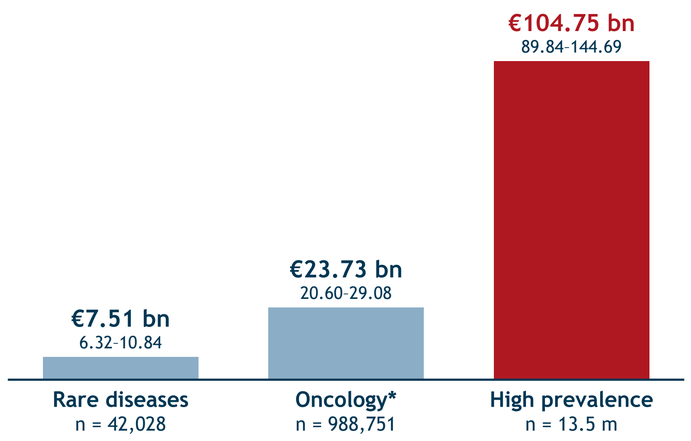
€ per patient per year, base case



Small figures = range, optimistic to pessimistic scenario. n = affected individuals in Germany, 2025. *Incl. 4 highest-mortality cancers, see 2.

2 Total cost by group

€ bn per year, base case



*7 cancer types with approved GCTs or advanced GCT development (€3.34 bn) plus breast, colorectal, prostate and pancreatic cancer (€20.4 bn); cost per patient prevalence-weighted.

3 Total burden over time

All groups, € bn, discounted at 3% p.a.

	Optimistic	Base case	Pessimistic
1 year	116.8	**136.0**	184.6
5 years	550.8	**641.5**	870.8
10 years	1,025.8	**1,194.8**	1,622.0

Pattern: highest cost per patient in rare diseases, highest total burden in high-prevalence diseases.

Plausibility: oncology estimate (€23.7 bn, 11 cancer types, incl. indirect costs) vs. €47.6 bn direct costs of all neoplasms in 2023[13].

Context: a UK assessment values wider GCT adoption at >£20 bn over 10 years – a complementary, benefit-side approach[14].

Conclusions & relevance

- A societal perspective reveals an economic burden of GCT-relevant diseases far larger than a direct-cost, single-therapy framing suggests.
- Reimbursement and policy discussions on GCTs should draw on societal BOI evidence, not only on AMNOG's direct-cost framework.
- Without linked, indication-level health data in Germany, model-based BOI extrapolation is a pragmatic and currently necessary tool – closing the data gap remains essential.

Limitations

- Intangible burden not monetised: totals understate the burden, most for low-utility (mainly rare) indications.
- Utilities largely international proxies (EQ-5D, TTO, simulated); few German studies.
- Rare diseases rest on two use-case cost profiles and sparse data; least uniform basis.
- Partial coverage (oncology ≈50–59% of cancer deaths; high prevalence ≈20–25% of people with a chronic condition) and ageing prevalence data: conservative lower bound.

www.bihealth.org

Contact:
Dr. Rebekka Mumm
rebekka.mumm@wig2.de

Dr. Joachim Weber
joachim.weber@bih-charite.de

References:

[1] Kranz P et al. (2024) Int J Technol Assess Health Care 40:e68
[2] O'Hara J et al. (2017) Orphanet J Rare Dis 12:106
[3] Rodriguez-Santana I et al. (2022) Orphanet J Rare Dis 17:150
[4] Duda H et al. (2020) J Clin Med 9:3408
[5] Andreas S et al. (2018) Lung Cancer 124:298–309
[6] McGuire A et al. (2015) J Med Econ 18:525–532
[7] RKI (2023) Kleinzellige und nicht-kleinzellige Lungenkarzinome. krebsdaten.de
[8] RKI (2025a) Zentrum für Krebsregisterdaten – Datenbankabfrage. krebsdaten.de
[9] Frielitz FS et al. (2022) Exp Clin Endocrinol Diabetes 130:614–620
[10] Hex N et al. (2012) Diabet Med 29:855–862
[11] Leśniowska J et al. (2014) Eur J Health Econ 15:653–660
[12] RKI (2025b) BURDEN 2020 – Krankheitslast in Deutschland und seinen Regionen
[13] Destatis (2025b) Krankheitskosten 2023. Pressemitteilung Nr. 293, 8 Aug 2025
[14] Sampson C et al. (2025) The value of cell and gene therapies to the UK economy. OHE